

PR 08-JAN-1996; 96US-0584043.  
XX (BAYU ) BAYLOR COLLEGE MEDICINE.  
PA Hauer J, Mims MP, Smith LC, Sparrow JT;  
PI WPI; 1997-372622/34.  
XX New lipophilic peptide-macromolecule complexes - used for the  
DR delivery of macromolecules to cells, particularly for gene therapy  
PT Disclosure; Page 16; 106pp; English.  
XX  
CC This sequence represents a delivery peptide that can be used in the  
CC peptide-macromolecule complex of the invention. The peptide-macromolecule  
CC complex of the invention is for delivering a macromolecule into a cell,  
CC and comprises a non-exchangeable lipophilic peptide (LP) comprising a  
CC delivery peptide associated with a lipid moiety, where the delivery  
CC peptide portion of the LP is complexed to the macromolecule. The  
CC complexes can be used for the delivery of macromolecules such as nucleic  
CC acids, proteins, oligonucleotides, lipids or carbohydrates. They can be  
CC used to treat diseases by enhancing delivery of specific nucleic acid to  
CC the appropriate targeted cells. They can also be used to create  
CC transformed cells as well as transgenic animals for assessing human  
CC disease in an animal model. They can also be used for livestock  
CC agricultural purposes. The complex is capable of transporting the  
CC macromolecule in a stable and condensed state and releasing the molecule  
CC into the cellular interior. The complex can bind with a cell surface  
CC receptor, lyse an endosome and target the nucleus of the cell.  
XX  
SQ Sequence 20 AA;  
Query Match 79.2%; Score 76; DB 18; Length 20;  
Best Local Similarity 75.0%; Pred. No. 7.5e-05;  
Matches 15; Conservative 5; Mismatches 0; Indels 0; Gaps 0;  
QY 1 GLPKALLKLLSLWLLILKA 20  
DB 1 GLPEALLELLSLWLLLEEA 20  
|||:||||:||||:||||:  
RESULT 14  
AAW24400  
ID AAW24400 standard; peptide; 20 AA.  
XX  
AC AAW24400;  
XX  
DT 26-SEP-1997 (first entry)  
XX  
DE JTS-1, a lytic agent used in nucleic acid delivery to cells.  
XX Nucleic acid transporter; gene therapy; binding complex; lysis agent;  
KW JTS-1; K8; alpha helix; endosome; lysosome; nucleus targeting.  
XX Synthetic.  
XX  
PH Key Location/Qualifiers  
FT Modified-site 1 /note= "acetylated"  
FT  
XX WO9640958-A1.  
XX  
XX 19-DEC-1996.  
XX  
XX 23-APR-1996; 96WO-US05679.  
XX  
XX 07-JUN-1995; 95US-0484777.  
XX (BAYU ) BAYLOR COLLEGE MEDICINE.  
XX  
XX Smith LC, Sparrow JT, Woo SL;  
XX WPI; 1997-052345/05.  
XX  
XX Nucleic acid transporter useful in gene therapy - contains binding  
XX complex associated with surface and nuclear ligands and lysis agent  
XX  
XX Disclosure; Page 92; 125pp; English.  
XX  
CC AAW24421-33 are modified versions of lytic peptides that were conjugated  
CC to a nucleic acid (NA) binding molecule (capable of both condensing  
CC and stabilizing the NA) to form a nucleic acid transporter system.  
CC The lysis agent forms an alpha-helical structure. The transporter  
CC system is used to deliver nucleic acid to a cell and for treating  
CC humans by gene therapy. By taking advantage of the characteristics of  
CC both the lysis agents and the binding molecules, delivery of the  
CC nucleic acid is enhanced. Specific lysis agents are capable of  
CC releasing the nucleic acid into the cellular interior from the endosome.  
XX Release is efficient without endosomal/lysosomal degradation. Once

PS Claim 5; Page 80; 125pp; English.  
XX  
CC AAW24400 is a lytic peptide agent that is conjugated to a nucleic acid  
CC (NA) binding molecule (capable of both condensing and stabilizing the  
CC NA) to form a nucleic acid transporter system. The lysis agent  
CC forms an alpha-helical structure. The transporter system is used to  
CC deliver nucleic acid to a cell and for treating humans by gene therapy.  
CC By taking advantage of the characteristics of both the lysis agents  
CC and the binding molecules, delivery of the nucleic acid is enhanced.  
CC Specific lysis agents are capable of releasing the nucleic acid  
CC into the cellular interior from the endosome. Release is efficient  
CC without endosomal/lysosomal degradation. Once released the binding  
CC complexes help target the nucleic acid to the nucleus.  
XX  
SQ Sequence 20 AA;  
Query Match 79.2%; Score 76; DB 18; Length 20;  
Best Local Similarity 75.0%; Pred. No. 7.5e-05;  
Matches 15; Conservative 5; Mismatches 0; Indels 0; Gaps 0;  
QY 1 GLPKALLKLLSLWLLILKA 20  
DB 1 GLPEALLELLSLWLLLEEA 20  
|||:||||:||||:||||:  
RESULT 15  
AAW24424  
ID AAW24424 standard; peptide; 20 AA.  
XX  
AC AAW24424;  
XX  
DT 26-SEP-1997 (first entry)  
XX  
DE Modified lytic peptide used in nucleic acid delivery to cells.  
XX Nucleic acid transporter; gene therapy; binding complex; lysis agent;  
KW JTS-1; K8; alpha helix; endosome; lysosome; nucleus targeting.  
XX Synthetic.  
XX  
PH Key Location/Qualifiers  
FT Modified-site 1 /note= "acetylated"  
FT  
XX WO9640958-A1.  
XX  
XX 19-DEC-1996.  
XX  
XX 23-APR-1996; 96WO-US05679.  
XX  
XX 07-JUN-1995; 95US-0484777.  
XX (BAYU ) BAYLOR COLLEGE MEDICINE.  
XX  
XX Smith LC, Sparrow JT, Woo SL;  
XX WPI; 1997-052345/05.  
XX  
XX Nucleic acid transporter useful in gene therapy - contains binding  
XX complex associated with surface and nuclear ligands and lysis agent  
XX  
XX Disclosure; Page 92; 125pp; English.  
XX  
CC AAW24421-33 are modified versions of lytic peptides that were conjugated  
CC to a nucleic acid (NA) binding molecule (capable of both condensing  
CC and stabilizing the NA) to form a nucleic acid transporter system.  
CC The lysis agent forms an alpha-helical structure. The transporter  
CC system is used to deliver nucleic acid to a cell and for treating  
CC humans by gene therapy. By taking advantage of the characteristics of  
CC both the lysis agents and the binding molecules, delivery of the  
CC nucleic acid is enhanced. Specific lysis agents are capable of  
CC releasing the nucleic acid into the cellular interior from the endosome.  
XX Release is efficient without endosomal/lysosomal degradation. Once

File copy